



DEPARTMENT OF HEALTH & HUMAN SERVICES

Public Health Service

March 24, 1987

National Institutes of Health
National Heart, Lung, and
Blood Institute
Bethesda, Maryland 20892

Dr. David A. Hamburg
President
Carnegie Corporation of N.Y.
437 Madison Avenue
New York, NY 10022

Dear Dr. Hamburg:

In reference to your letter of March 18, I have written to Dr. Benedict (copy attached). It is estimated there are 20 to 40,000 α 1-antitrypsin deficient individuals in the United States with probably equal (or more) in Europe. Needless to say, since we developed this approach to therapy, we have been deluged with requests to admit patients into the study.

As I outlined in the letter to Dr. Benedict, the major obstacle to having her husband participate in such a study is the fact that the Food and Drug Administration has limited the use of the material to the United States. For the first 18 months of our evaluation, they had limited the use of the material only to being administered at our Institution; they have now allowed us to give it out to physicians around the United States. This may be insurmountable for Dr. Benedict's husband. However, we would be pleased to evaluate him if they could come to the United States. Then the various aspects of participation in such studies could be discussed in detail.

If they could arrange to have the clinical information from their physician sent to me, we could make arrangements within a few weeks to have Dr. Dr. Benedict's husband evaluated.

I am enclosing some articles that I sent to Dr. Benedict; I thought these might be of interest. I am also enclosing an article relating to the therapy itself; the latter is scheduled to be published in the New England Journal of Medicine later next month.

I do not know whether we can be helpful to Dr. Benedict's husband but we will do everything possible.

Sincerely yours,

Ronald G. Crystal, M.D.
Chief, Pulmonary Branch
National Heart, Lung, and Blood Institute

cc: Joshua Lederberg

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